



# **National Plans for Rare Diseases**

## **The French plan 2005-2008**

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# Political Context

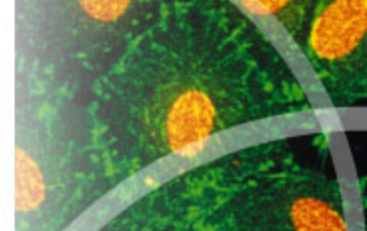
- Public Health Law of 9 August 2004
  - 100 defined targets
  - Rare Diseases among the **5 Top Priorities**
- Previous initiatives:
  - Orphan Drug Office established in 1995
  - Orphanet established in 1996
  - Eurordis launched in 1997
  - Rare Disease Platform established in 2001

# Ten strategic priorities and 100 Millions Euros

- 1- Increase knowledge of the epidemiology of RD
- 2- Recognise the specificity of RD
- 3- Develop information for all stakeholders
- 4- Train professionals
- 5- Organise screening and access to diagnostic tests
- 6- Improve access to treatment and healthcare provision
- 7- Continue effort in favour of Orphan Drugs
- 8- Respond to social needs and support patients' organisations
- 9- Promote research and innovation
- 10- Develop national and European partnership

- > Information about a disease
- > Alphabetical list
- > Search by clinical sign
- > Emergency guidelines
- > Patient encyclopaedia
- > Professional encyclopaedia
- > About Rare Diseases

**Provide** adapted services  
for the rare diseases community



[About Orphanet](#) | [Quality charter](#)  
[Register your activity](#)

## RESOURCES DIRECTORY

- > Clinics
- > Reference centres
- > Diagnostic tests
- > Research projects
- > Registries / databases
- > Professionals
- > Patient organisations
- > EuroGentest
- > Register your activity

## ORPHAN DRUGS

- > Information about an orphan drug
- > Clinical trials
- > Register your clinical trial
- > About orphan drugs

## MEDIA

- > About Orphanet
- > About orphan drugs
- > About Rare diseases
- > Press Releases

## EDUCATIONAL TOOLS

- > Training sessions
- > Orphanet Report Series

Languages : [Français](#) | [English](#) | [Español](#) | [Deutsch](#) | [Italiano](#)

### Services for professionals

- > Professional encyclopaedia
- > Search by clinical sign
- > Emergency guidelines
- > Orphanet Journal Of Rare Diseases
- > Newsletters
- > Powerpoints
- > Register your activity
- > OrphanXchange

### Services for patients

- > Patient encyclopaedia
- > Information about a disease
- > Patient organisations
- > Clinics
- > Participate in clinical research
- > Contact other patients / families
- > Training sessions
- > Newsletters

### Services for organisations

- > Patient organisations
- > Register your organisation
- > Website creation and hosting assistance

### Services for industry

- > Orphan drugs
- > Clinical trials
- > Register your activity
- > OrphanXchange
- > Newsletters

## NEWS

[Press release](#)

[European public consultation on rare diseases](#)

[Rare disease day](#)

## EVENTS

[Myology 2008](#)

[First International Meeting of Moebius Syndrome](#)

[Other events \(pdf\)](#)

## ORPHANET DOCUMENTS

- > [Prevalence of rare diseases \(pdf\)](#)
- > [Marketing authorised orphan drugs in Europe \(pdf\)](#)

## OTHER DOCUMENTS

- > [Rare Diseases: Europe's Challenges \(pdf\)](#)
- > [EC Rare Disease webpages](#)
- > [French National Plan for Rare Diseases \(pdf\)](#)

## ORPHANET IN COUNTRIES

[Armenia](#)  
[Austria](#)  
[Belgium](#)  
[Bulgaria](#)  
[Croatia](#)  
[Cyprus](#)

[Estonia](#)  
[Finland](#)  
[France](#)  
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# Achievements: Epidemiology

- In the field of registries
  - Establishment of a national committee in charge of registries for rare diseases
  - Joint call for proposals (public health + research)
  - Designation of registries but without funding
- In the field of information systems
  - Obligation for the centres of reference to collect clinical data on all rare diseases seen at their centre
  - Budget allocated to this activity
  - No harmonisation of systems (3 main tools)
  - Tools developed by Orphanet

# Achievements: Specificity of RD

- Development of national protocols for diagnosis and care by the HTA agency (HAS)
- Reimbursement of drugs and devices with no official indication for RD, or outside the usual reimbursement scope (on request of centres of reference or patients' organisations)
- Reimbursement of transport costs to attend clinics at centres of reference
- Establishment of a RD office at the national health insurance agency to deal with all problems and harmonise practices in the regions

# Achievements: Information

- New services at Orphanet
  - Encyclopaedia for patients in French
  - Emergency guidelines
  - Search by sign facility
  - Introduction of classifications to improve search facility
  - Portal accessible to disabled
  - Publication of a book distributed to 10 000 professionals
- Emergency cards
  - Produced by the Ministry / distributed by the centres of reference
- Support to Helpline



# Achievements: Training

- Introduction of two hours in the cursus of medical students:
  - existence of RD + how to access relevant information
- Optional modules for medical students
  - 30 hours course
- Similar measures for non MD health professionals



# Achievements: Screening/Testing

- Screening
  - Nothing done for political reasons
  - Project: establish a committee to assess population screening proposals and review outcomes
- Testing
  - To improve availability of diagnostic tests
  - Organisation of networks of laboratories (oncogenetics, neurogenetics, mental retardation, neurosensory genetics...)
  - Reference laboratories
  - Funding of testing activities (20 Millions)

# Achievements: Healthcare provision

- Establishment of centres of reference
  - Establishment of a national committee
  - Through annual call for proposals
  - National coverage / based on scientific expertise + volume of activity + real 5 year plan to improve care
  - 132 centres approved and funded (over 100 M Euros already)
    - 200 new positions for MD + 200 new positions for non MDs: permanent
  - Mission of expertise, information, training and research / self assessment at 3 years / external assessment at 5 years
- Establishment of centres of competence
  - At the request of patients' organisations
  - At regional level
  - Network common to several centres of reference sometimes

# Achievements: Support orphan drugs

- Preservation of existing situation
  - Exemption for the promoters of OD with respect to taxes and payments due
  - OD on the list of innovative and expensive health products to be systematically covered
  - Prevention of unavailability of marketed OD
  - « Autorisation temporaire d'utilisation » scheme to be continued

# Achievements: Social needs

- Coordination between Plan for Rare Diseases and Plan for Disabled people:
  - recognition of the additional burden attached to RD
- Publication of a brochure on rights and opportunities for patients with RD
  - By Orphanet
  - Available on the frontpage of the website
  - Included in the encyclopaedia for the patients
  - Distribution of the Orphanet book to all institutions for disabled

# Achievements: Promotion of Research

- Annual call for proposals for preclinical research
  - GIS-Institut des maladies rares
  - Networks, registries, platforms and all types of research projects
  - 30 M Euros
- Annual call for proposals for clinical research
  - For hospital-based teams
  - Academic trials and clinical research in general
  - 26 M Euros
- Annual joint call with some other EU countries
  - E-Rare: 9 M Euros first year (2.4 from France)

# Achievements: Partnerships

- National coordination of all partners
  - Follow-up committee
- Support to the Rare Diseases Platform in Paris
  - GIS maladies rares- Orphanet – Alliance Maladies Rares – Eurordis – Helpline
  - Hosting of 200 meetings per year
  - Communication
- Contribute to European policy
  - Orphanet in 38 countries
  - Rare Diseases Task Force secretariat
  - Eurordis
  - Proactive Ministry of Health / contribution to the EC Communication

# CONCLUSION

- Assessment of the national plan :
  - High level Public Health Committee
  - December 2008
- Difficult to document yet the objective effects
- Very positive feeling of stakeholders
- Support of ongoing initiatives in the future
- Lessons to be learned